# Expanded Capacity AAV Retinal Gene Therapy Enabled by Efficient RNA-Joining Technology

## Grant Award Details

Expanded Capacity AAV Retinal Gene Therapy Enabled by Efficient RNA-Joining Technology

**Grant Type:** Quest - Discovery Stage Research Projects

**Grant Number:** DISC2-14187

**Investigator:**

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|---|---|
| **Name:** | Lukas Bachmann |
| **Institution:** | Vertuis Bio, Inc. |
| **Type:** | PI |

**Award Value:** $1,446,000

**Status:** Pre-Active

## Grant Application Details

**Application Title:** Expanded Capacity AAV Retinal Gene Therapy Enabled by Efficient RNA-Joining Technology

**Public Abstract:**

**Research Objective**

A novel AAV-based gene therapy candidate for use in treatment of inherited ocular disease.

**Impact**

AAV vectors have a limited cargo capacity (<5kb), preventing their use in the treatment of many untreatable genetic diseases caused by large genes.

**Major Proposed Activities**

- In vitro optimization and selection of gene therapy candidates
- In vivo testing to establish efficacy of gene therapy candidates
- Demonstration of disease-modifying activity in mouse genetic model of ocular disease
- Evaluation of safety for a dual vector AAV approach
- Testing activity in human photoreceptors

**Statement of Benefit to California:** Of the 280 genetic mutations that cause inherited retinal disease (IRD), there is an approved treatment for just one. Our innovative technology provides a solution for challenges encountered by conventional AAV-based gene therapy approaches. Because our technology is agnostic to gene, the advancement of this therapy will be an important step forward for developing treatment for a wide variety of genetic diseases, including those that impact the diverse California population.

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